NCATS National Center for Advancing Translational Sciences
Catalyzing Innovation

Discovering New Therapeutic Uses for Existing Molecules
A Pilot NIH-Industry Program

Program Overview

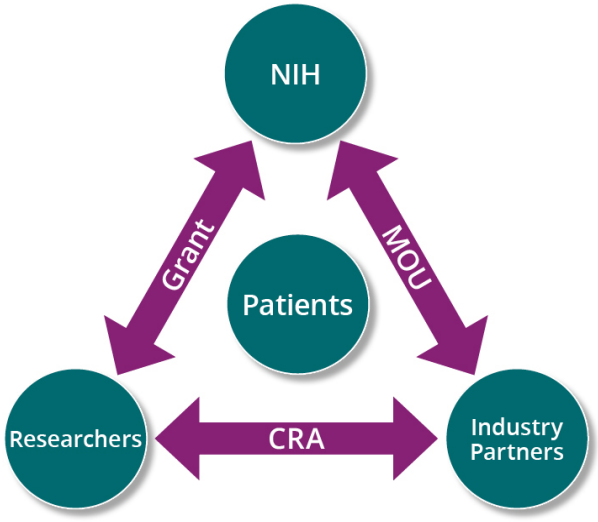
Therapeutic development is a costly, complex and time-consuming process. In recent years, researchers have succeeded in identifying the causes of nearly 4,500 diseases. It has proven difficult, however, to turn such knowledge into new therapies; effective treatments exist for only about 250 of these diseases.

To help combat these challenges, the National Institutes of Health (NIH) recently unveiled its Discovering New Therapeutic Uses for Existing Molecules collaborative pilot program, which aims to tackle an urgent need that is beyond the scope of any one organization or sector. Administered by the National Center for Advancing Translational Sciences (NCATS), the Therapeutics Discovery initiative matches researchers with a selection of molecular compounds from industry to test ideas for new therapeutic uses, with the ultimate goal of identifying promising new treatments for patients.

NCATS has collaborated initially with Abbott, AstraZeneca, Bristol-Myers Squibb Company, Eli Lilly and Company, GlaxoSmithKline, Janssen Pharmaceutical Research & Development, L.L.C, Pfizer, and Sanofi, which collectively have agreed to make 58 compounds available for the pilot program. The compounds have undergone significant research and development by industry, including safety testing in humans, providing a strong starting point for scientists and permitting the process to move more rapidly.

How the Therapeutics Discovery Program Works

In Fiscal Year 2013, NCATS will provide up to $20 million for the Therapeutics Discovery program, which will fund two- to three-year staged, cooperative agreement research grants. In June 2012, NIH issued a Funding Opportunity Announcement (FOA) asking U.S. investigators for short applications that describe how investigators would explore specific hypotheses about how a compound available through the pilot might be useful in a specific disease area. Summary information about the compounds is available at ncats.nih.gov/therapeutics-directory.html.



In July 2012, researchers can apply to the pilot program through the FOA. These pre-applications, due August 14, will undergo review by outside experts, and successful applicants will be notified that they may submit a full application. Applicants may then sign confidential disclosure and collaborative research agreements with the industry partner to obtain detailed information about the specific compound of interest in order to develop a full application.

Full applications, due in December 2012, then will undergo NIH peer review. Funding selections will follow, and cooperative agreement research grants will be awarded by summer 2013.

For two-stage (UH2/UH3) awards, the first phase is expected to focus on pre-clinical and clinical studies to explore the feasibility of testing the compound for a new therapeutic use. If specific milestones are met, NIH will provide funding for the UH3, a proof-of-concept clinical trial for up to two years. Some applications may request support for only the UH3 proof-of-concept trial for up to two years.

The pilot also is intended to test the utility of the template agreements by reducing the negotiation time that otherwise could delay the research. In addition, the program is expected to generate exceptional science and some candidate compounds that will advance for further development.

About NCATS and NIH

NCATS aims to catalyze the generation of innovative methods and technologies that will enhance the development, testing and implementation of diagnostics and therapeutics across a wide range of human diseases and conditions. By improving the process by which diagnostics and therapeutics are developed, NCATS strives to make translational science more efficient, less expensive and less risky. In this way, NCATS is complementing — not competing with — the work of the private sector and other NIH Institutes and Centers. Visit ncats.nih.gov to learn more.

NIH, the nation's medical research agency, includes 27 Institutes and Centers and is a component of the U.S. Department of Health and Human Services. NIH is the primary federal agency conducting and supporting basic, clinical and translational medical research, and is investigating the causes, treatments and cures for both common and rare diseases. Visit www.nih.gov.

For More Information

Contact
NCATS Communications at 301-435-0888 or info@ncats.nih.gov.

Website
Visit NCATS online at ncats.nih.gov/therapeutics.html.

Funding Information
Review the funding announcements at ncats.nih.gov/research/reengineering/rescue-repurpose/therapeutic-uses/funding.html.

Frequently Asked Questions
To view answers to frequently asked questions, visit ncats.nih.gov/therapeutics-faq.html.